

Research Participation in Huntington's Disease: Perceptions and Experiences of the Dutch Community

Filipa Júlio^{1,2}, Ruth Blanco^{1,3}, José Perez Casanova^{1,3}, Barbara D'Alessio^{1,4}, Beatrice De Schepper^{1,5}, Dina De Sousa^{1,6}, Paul De Sousa^{1,6}, Cristina Ferreira^{1,2}, Hans Gommans^{1,7}, Rob Haselberg^{1,7}, Emilie Hermant^{1,8}, Danuta Lis^{1,9}, Sabrina Maffi¹⁰, Svein Olaf Olsen^{1,11}, Marios Papantoniou^{1,12}, Ferdinando Squitieri¹⁰, Marina Tretyakova^{1,13}, Zaynab Umakhanova^{1,13}, Vladimír Václavík^{1,14}, Michaela Winkelmann^{1,15}, and Astri Arnesen^{1,11} on behalf of the European Huntington Association

1 European Huntington Association – Belgium, 2 Associação Portuguesa dos Doentes de Huntington – Portugal, 3 Asociación Corea de Huntington Española – Spain, 4 Fondazione Lega Italiana Ricerca Huntington – Italy, 5 Huntington Liga – Belgium, 6 Scottish Huntington's Association – Scotland, 7 Vereniging Van Huntington – The Netherlands, 8 L'Association Huntington France – France, 9 Polskie Stowarzyszenie Choroby Huntingtona – Poland, 10 Huntington and Rare diseases Unit, IRCCS Casa Sollievo della Sofferenza Research Hospital – Italy, 11 Landsforeningen for Huntingtons Sykdom – Norway, 12 Huntington's Disease Association of Cyprus – Cyprus, 13 Orphan People – Russia, 14 Spoločnosť Pre Pomoc Pri Huntingtonovej Chorobe – Slovakia, 15 Deutsche Huntington Hilfe E.V. – Germany



filipa@eurohuntington.org

Introduction

Despite the several progresses in Huntington's disease (HD) research over the last years [1], effective disease-modifying therapies that can stop HD before the onset of any disabling symptoms are still lacking [2]. Because clinical progresses imply an active, informed, and lasting commitment from HD families with research, they should be heard in this process [3]. Specifically, persons at risk for HD whose genetic status is unknown (HdRisk) and persons that tested positive for HD and are in a so-called premanifest or prodromal disease stage (PreHD) should be more engaged in studies and trials. Therefore, our main goal was to determine which factors affect the willingness of persons with HdRisk and PreHD to take part in studies. A comprehensive presentation of this work can be found elsewhere [4]. Here, our aim was to investigate the view of the Dutch HdRisk and PreHD communities regarding research participation.

Methods

The European Huntington Association (EHA) created an anonymous online survey to collect information about the perceptions and experiences of research participation among persons with HdRisk and PreHD across Europe (Figure 1). The survey was created through the SurveyMonkey platform [5], contained twelve questions and took around eight minutes to complete. The survey was translated and made available in 12 languages: Dutch (with variants for use in Belgium and The Netherlands), English, French, German, Italian, Norwegian, Polish, Portuguese, Russian, Slovakian, and Spanish. Questions assessed research experience and knowledge, sources of information about research, reasons for involvement and noninvolvement in research, and factors preventing and facilitating study participation. The Dutch survey responses were examined and compared with the global survey responses.

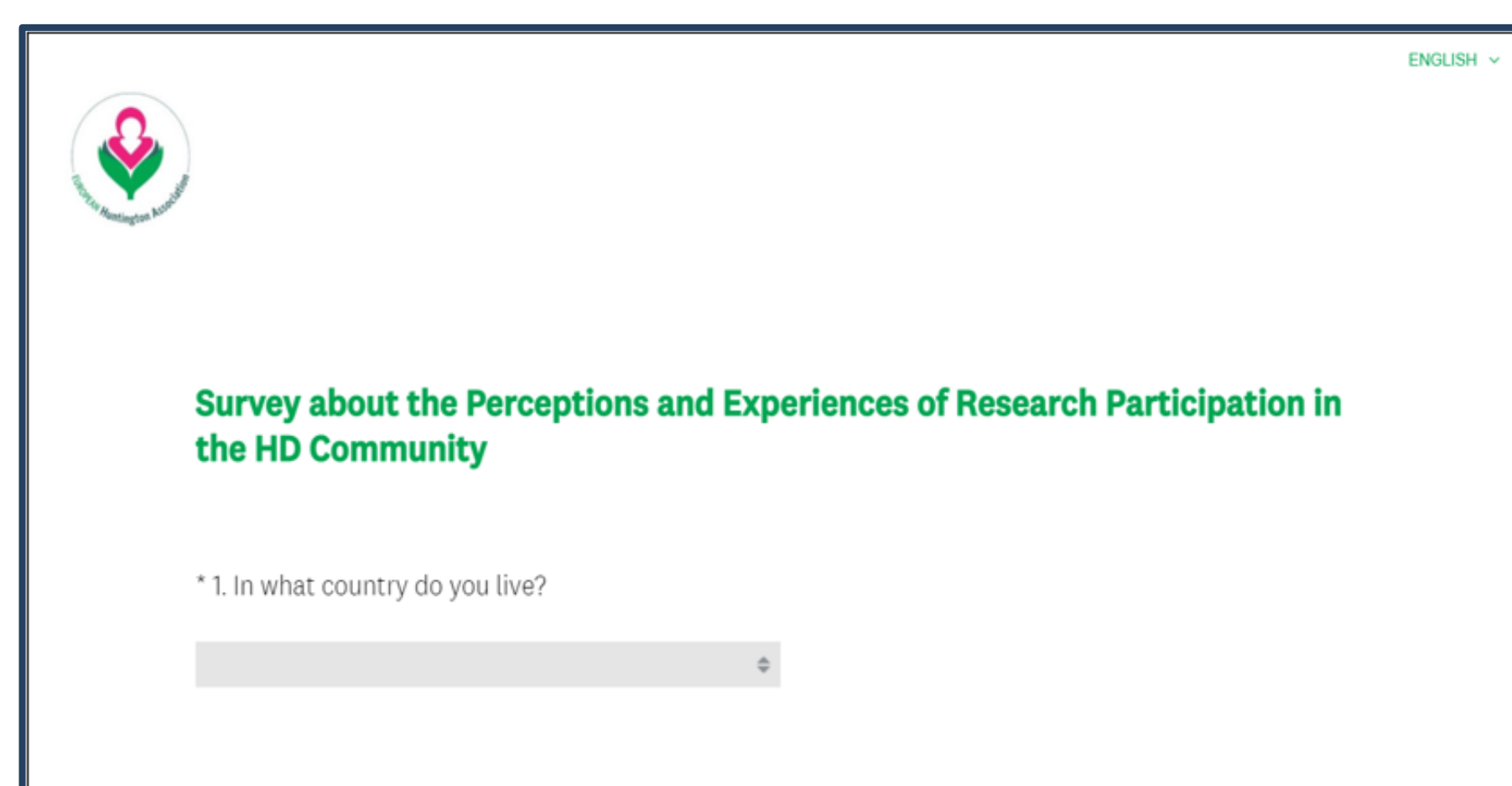


Figure 1 EHA Online Survey

Results

The survey was filled by 525 persons with HD Risk and PreHD from 27 countries. We found that the Dutch survey respondents (DSR, n=87) were older and less educated than the global survey respondents (GSR, n=438), and relied more on their family and on television to get research information - see Table 1 and Table 2. Overall, the reasons and factors enabling study involvement were less important for the DSR than the GSR - see Figure 2 a) and Figure 3 a). The need to travel to the study site and invasive procedures, but not care assistance, were greater obstacles to participation for the DSR than the GSR - see Figure 2 b). Importantly, the information and support provided by others had a reduced influence on the DSR study engagement compared to GSR - see Figure 2 a) and b) and Figure 3 a). Lastly, negative emotions were less important obstacles to DSR research participation - see Figure 3 b).

	Demographics		Chi-Square Dutch vs Total χ^2 (p-value)
	Dutch n= 87 %	Total n= 438 %	
HD Status			
At risk for HD	49.4	50.1	0.019 (0.891)
Premanifest/Prodromal HD	50.6	50	
Gender			
Female	71.3	71.6	0.006 (0.936)
Male	28.7	28.4	
Age Interval (years)			
18 to 24	8	8.6	0.037 (0.848)
25 to 34	29.9	29.5	0.007 (0.936)
35 to 44	27.6	33.1	1.453 (0.228)
45 to 54	19.5	18.3	0.110 (0.740)
55 to 64	13.8	8.4	3.978 (0.046) *
65 to 74	1.1	1.5	0.097 (0.755)
75 or older	0	0.6	0.599 (0.439)
Education Level			
Did Not Attend School	0	0	-
Completed Primary Education	1.1	1	0.043 (0.836)
Completed Secondary Education	35.6	20.4	14.948 (< 0.001) **
Graduated from High School	16.1	28.4	7.748 (0.005)
Graduated from College	41.4	26.5	11.898 (0.001)
Completed Graduate School	5.7	23.8	18.754 (< 0.001) **
Previous HD Research Experience			
Yes	24.1	31.2	2.447 (0.118)
No	75.9	68.8	

Table 1 - Demographic characteristics of survey respondents * statistically significant $p \leq 0.05$ ** statistically significant $p \leq 0.01$

	Knowledge and Information about HD Research		Chi-Square Dutch vs Total χ^2 (p-value)
	Dutch n= 87 %	Total n= 525 %	
Knowledge about HD Research			
Not Good	3.4	5.3	0.734 (0.392)
Should be Better	28.7	33.3	0.992 (0.319)
Satisfactory	29.9	24.6	1.589 (0.208)
Good	26.4	28.6	0.233 (0.629)
Excellent	11.5	7.8	1.967 (0.161)
Do not want to know about HD research	0	0.4	0.399 (0.528)
Sources of Information about HD Research			
Internet	77	78.5	0.132 (0.716)
Television	12.6	4	20.289 (< 0.001) **
Press/Newsletters/Flyers/Booklets	16.1	19.8	0.907 (0.341)
HD Associations and/or Support Groups	51.7	52	0.003 (0.955)
Health Care Professionals	19.5	25.5	1.964 (0.161)
Family Members	44.8	25.3	20.951 (< 0.001) **
Not interested in HD research information	1.1	0.4	1.623 (0.203)

Table 2 – Level of Knowledge and Sources of Information about HD Research

* statistically significant $p \leq 0.05$ ** statistically significant $p \leq 0.01$

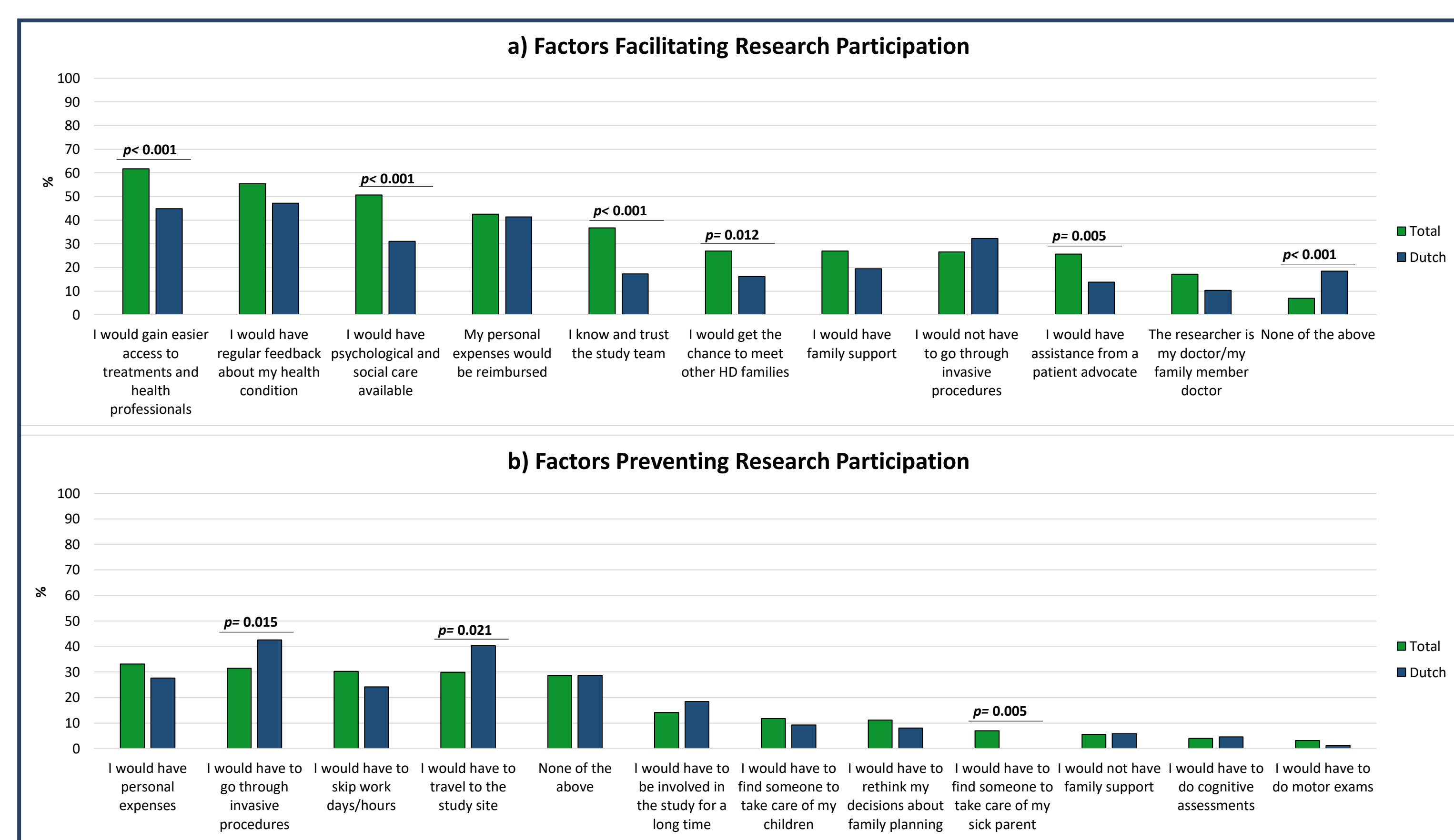


Figure 2 – Moderators of Research Participation: Factors Facilitating (a) and Preventing (b) Research Participation (percentage of participants that selected each factor)

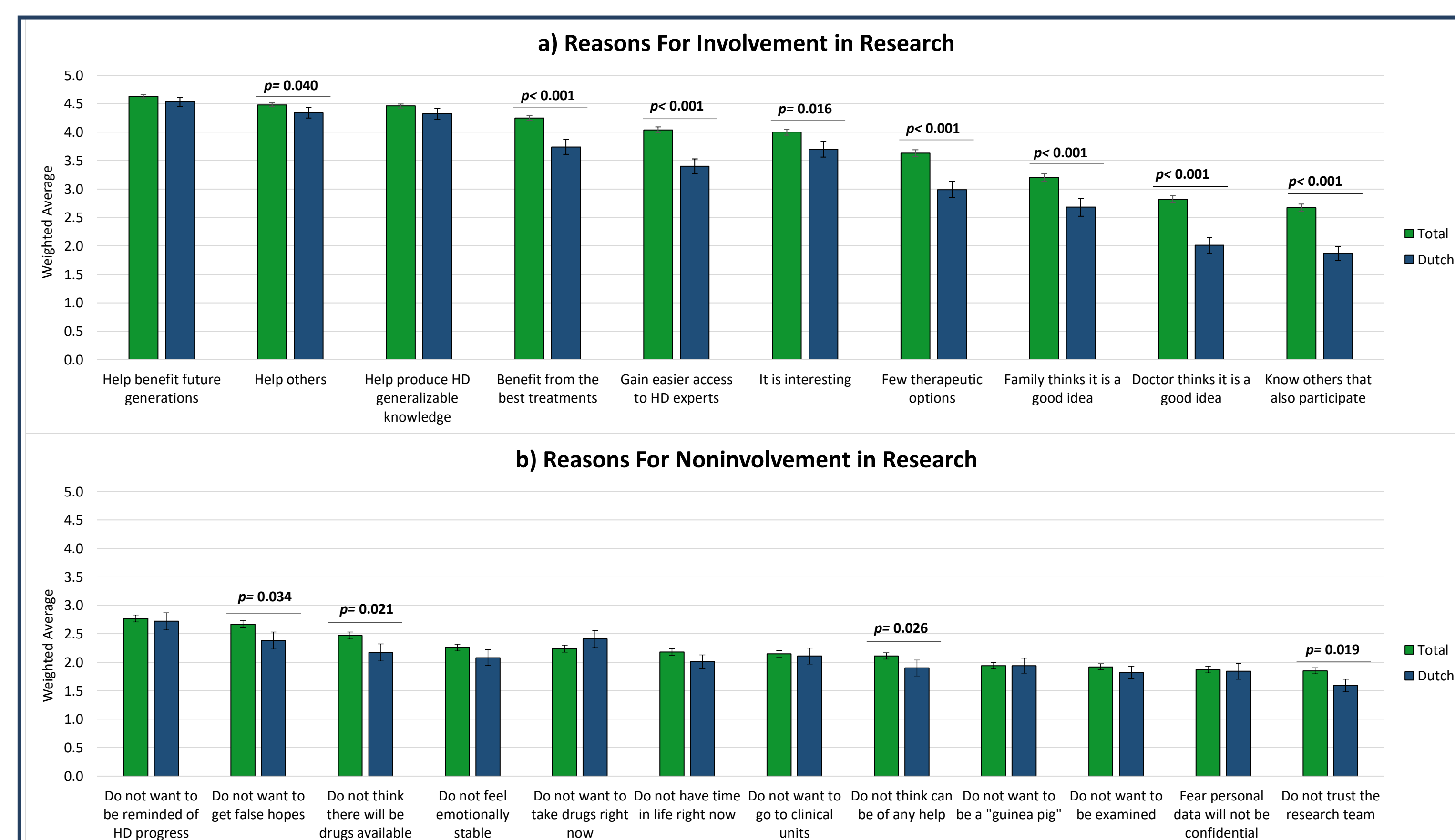


Figure 3 Motivators for Research Participation: Reasons for Involvement (a) and Noninvolvement (b) in Research (weighted average score of the importance of each reason on a range 1-5)

Key Findings and Conclusions

- The Dutch survey responses generally overlap the global survey results
- Motivation to take part in studies is high, despite limited research experience and literacy
- This motivation is strongly influenced by tangible factors such as site distance and invasiveness of procedures, but not by care burden
- This motivation is less affected by external factors and emotional status

Our findings suggest that:

- ➔ the Dutch HdRisk and PreHD communities consider their formal/informal healthcare assistance model to be effective
- ➔ the Dutch HdRisk and PreHD communities present an intrinsic motivation to participate in research
- ➔ the information and motivation of the Dutch HdRisk and PreHD communities may be potentially enhanced through family education and multimedia campaigns

References:
 1 Dash, D., & Mestre, T. A. (2020). Therapeutic Update on Huntington's Disease: Symptomatic Treatments and Emerging Disease-Modifying Therapies. *Neurotherapeutics: the Journal of the American Society for Experimental Neurotherapeutics*, 17(4), 1645–1659. <https://doi.org/10.1007/s13311-020-00891-w>
 2 Shoulson, I., & Young, A. B. (2011). Milestones in huntington disease. *Movement disorders: official journal of the Movement Disorder Society*, 26(6), 1127–1133. <https://doi.org/10.1002/mds.23685>
 3 Price, A., Albarqouni, L., Kirkpatrick, J., Clarke, M., Lew, S. M., Roberts, N., & Burls, A. (2018). Patient and public involvement in the design of clinical trials: An overview of systematic reviews. *Journal of evaluation in clinical practice*, 24(1), 240–253. <https://doi.org/10.1111/jep.12805>
 4 Júlio, F., Blanco, R., Casanova, J. P., D'Alessio, B., De Schepper, B., De Sousa, D., De Sousa, P., Ferreira, C., Gommans, H., Haselberg, R., Hermant, E., Lis, D., Maffi, S., Olsen, S. O., Papantoniou, M., Squitieri, F., Tretyakova, M., Umakhanova, Z., Václavík, V., Winkelmann, M., ... On Behalf Of The European Huntington Association (2021). Perceptions about Research Participation among Individuals at Risk and Individuals with Premanifest Huntington's Disease: A Survey Conducted by the European Huntington Association. *Journal of personalized medicine*, 11(8), 815. <https://doi.org/10.3390/jpm11080815>
 5 Survey Monkey. Available online: <https://www.surveymonkey.com/> (accessed on 3 September 2021)